



Our STN: BL 125694/444

SUPPLEMENT APPROVAL

July 26, 2024

Novartis Gene Therapies, Inc.
Attention: Lisa Krueger, PharmD
2275 Half Day Road, Suite 200
Bannockburn, IL 60015

Dear Dr. Krueger:

We have approved your request received January 26, 2024, to supplement your Biologics License Application (BLA) submitted under section 351(a) of the Public Health Service Act for onasemnogene abeparvovec-xioi (ZOLGENSMA®) to update the Adverse Reactions section of the US Prescribing Information to include the observed incidence of transaminase elevations and transient thrombocytopenia in patients with SMA weighing ≥ 8.5 kg, based on Study COAV101A12306 (NCT04851873) safety results.

LABELING

We hereby approve the draft content of labeling for the Package Insert submitted under amendment 7 via eCTD Sequence 0348, dated July 25, 2024.

CONTENT OF LABELING

As soon as possible, but no later than 14 days from the date of this letter, please submit the final content of labeling (21 CFR 601.14) in Structured Product Labeling (SPL) format via the FDA automated drug registration and listing system, (eLIST) as described at <http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm>. Content of labeling must be identical to the Package Insert submitted on July 25, 2024. Information on submitting SPL files using eLIST may be found in the guidance for industry *SPL Standard for Content of Labeling Technical Qs and As* at <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM072392.pdf>.

The SPL will be accessible via publicly available labeling repositories.

All final labeling should be submitted as Product Correspondence to this BLA, STN BL 125694/0 at the time of use and include implementation information on Form FDA 356h.

ADVERTISING AND PROMOTIONAL LABELING

You may submit two draft copies of the proposed introductory advertising and promotional labeling with Form FDA 2253 to the Advertising and Promotional Labeling Branch at the following address:

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71–G112
Silver Spring, MD 20993-0002

You must submit copies of your final advertising and promotional labeling at the time of initial dissemination or publication, accompanied by Form FDA 2253 (21 CFR 601.12(f)(4)).

All promotional claims must be consistent with and not contrary to approved labeling. You should not make a comparative promotional claim or claim of superiority over other products unless you have substantial evidence or substantial clinical experience to support such claims (21 CFR 202.1(e)(6)).

We will include information contained in the above-referenced supplement in your BLA file.

Sincerely,

‘Lola Fashoyin-Aje, MD, MPH
Acting Director
Division of Clinical Evaluation General Medicine
Office of Clinical Evaluation
Office of Therapeutic Products
Center for Biologics Evaluation and Research